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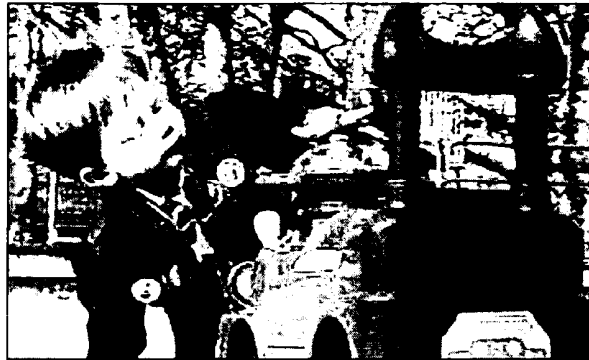
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## 'Bubble boy' saved by gene therapy



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Rhys can now play outside

In one of the first treatments of its kind, UK doctors have used gene therapy to cure a toddler of a potentially fatal disorder.

Scientists at Great Ormond Street Hospital in London successfully treated 18-month-old Rhys Evans, who had a condition preventing him from developing an immune system.

The problem, called severe combined immunodeficiency (SCID) is caused by a single mutated gene, and meant that he had to live in sterile conditions or risk picking up a life-threatening infection.

Rhys, from Treharris, nr Cardiff, had spent much of his life in hospital prior to the treatment.

Only one in between 50,000 and 100,000 births in the UK is affected by the disorder.

However, following the ground-breaking treatment, which adds in a correctly-functioning version of the faulty gene, it is hoped that he will be able to lead a

**After his gene therapy, he was running around at home - he's a normal little boy now**

**Dr Adrian Thrasher, Great Ormond Street Hospital**

**WATCH/LISTEN**

**The BBC's Karen Allen**

"He was born without an immune system"

**Rhys Evans' consultant Dr Adrian Thrasher**

"We know what the genetic defect in his bone marrow is"

**Professor Christine Kinnon, Great Ormond Street**

"There are other immune diseases which will be applicable to this treatment"

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**Internet links:**

normal life.

And doctors are hopeful that children with other similar genetic disorders could benefit in a similar way.

Gene therapy for SCID - often dubbed the "baby in a bubble" syndrome - has only been successful in two other cases, treated at a Paris Hospital in 2000.

### Gene therapy hurdles

In general, attempts to use gene therapy in a wide variety of medical fields have had only limited success so far.

This is because it has proved difficult to get the therapy to the cells which need it.

However, the Great Ormond Street team, led by Dr Adrian Thrasher, is now moving on to tackle other similar disorders caused by a single gene defect.

Dr Thrasher told the BBC: "We're very excited by this - he was incredibly sick, with a nasty pneumonia, a life threatening infection.

**Since they cracked the genetic code and found the gene, they can work miracles**

"After his gene therapy, he was running around at home - he's a normal little boy now."

**Marie Evans, Rhys' mother**

Rhys' mother Marie said that she had been restricted to fortnightly visits because Rhys had to stay at Great Ormond Street while she returned home.

She said: "It is difficult to look at what we have been through - we see him now, playing with other children, and it's just amazing.

"Since they cracked the genetic code and found the gene, they can work miracles."

**No donor**

Great Ormond Street/Institute of Child Health

SCID homepage

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Rhys needed to be kept in a sterile environment

Rhys, a matching donor could not be found.

Instead, Dr Thrasher's team took bone marrow from the boy, then used a virus to carry a new version of the gene into immune cells from the marrow.

This was then reimplanted into the patient, where it gradually began to generate further cells to pass into the bloodstream and protect him from infection.

Now he has a normal count of immune cells for a child of his age, and doctors are hopeful this will continue.

This particular type of SCID, called "X-linked" SCID, affects only boys.

The scientists now want to try to correct an illness called chronic granulomatous disorder (CGD), which again involves a single defective gene.

The success is the first for the newly-launched gene therapy laboratory at Great Ormond Street, which was partly funded by the trust's "Jeans for Genes" appeal.

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